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**DEPARTMENT OF HEALTH AND HUMAN SERVICES**

**National Institutes of Health**

**Government-Owned Inventions; Availability for Licensing**

**AGENCY:** National Institutes of Health, HHS.

**ACTION:** Notice.

**SUMMARY:** The inventions listed below are owned by an agency of the U.S. Government and are available for licensing in the U.S. to achieve expeditious commercialization of results of federally-funded research and development.

**FOR FURTHER INFORMATION CONTACT:** Licensing information may be obtained by emailing the indicated licensing contact: Michael Shmilovich; National Heart, Lung, and Blood, Office of Technology Transfer and Development Office of Technology Transfer, 31 Center Drive Room 4A29, MSC2479, Bethesda, MD 20892-2479; telephone: 301-402-5579. A signed Confidential Disclosure Agreement may be required to receive any unpublished information.

**SUPPLEMENTARY INFORMATION:** Technology description follows.

### **Sickle Cell Anemia Treatment through RIOK3 inhibition**

Beta-globinopathy is a common inherited single-gene disorder of beta-globin synthesis that results in an abnormal structure of one globin chain of the hemoglobin molecule. Common hemoglobinopathies include sickle-cell disease and beta-thalassemia. The efficacy of bone marrow transplantation is limited due to high cost and the requirement for HLA-matched donors. Increasing fetal hemoglobin expression above a certain threshold is potentially curative in the beta-globinopathies. The inventors identified Rio-Kinase 3 (RIOK3) as a key negative regulator of fetal hemoglobin expression in primary human erythroid progenitor cells. Their work shows that lentiviral mediated shRNA knockdown of RIOK3 in primary human erythroid progenitor cells increased fetal hemoglobin expression above 55% of total b-like globin expression, thus, RIOK3 is a promising novel therapeutic target to increase fetal hemoglobin expression.

### **Potential Commercial Applications:**

Inhibition of RIOK3 through genetic manipulation or by using orally administered kinase inhibitors will be a novel and cost-effective treatment strategy in beta-globinopathies.

### **Development Stage:**

The potential clinical use of this novel invention will depend on collaborating with interested companies for efficiently inhibiting RIOK3 through (1) designing lentiviral vectors encoding shRNA to RIOK3, (2) gene editing using endonucleases such as

CRISPR/Cas9 and (3) by developing orally administered RIOK3 specific kinase inhibitor drugs.

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**Intellectual Property:** HHS Reference No. E-200-2018; U.S Provisional Patent Application 62/756,497 filed November 6, 2018.

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